

# PODIUM SESSION I: ALTERNATIVE VIEWS IN CANCER OUTCOMES RESEARCH TO COLLECT ALL BENEFITS

## CN1

### MEAN VERSUS MEDIAN OVERALL SURVIVAL (OS) FOR DESCRIBING VALUE OF NEW CANCER THERAPIES: A CASE STUDY

Davies A<sup>1</sup>, Briggs A<sup>2</sup>, Wagner S<sup>3</sup>, Kotapati S<sup>4</sup>, Schneider J<sup>5</sup>, Ebeid O<sup>5</sup>, Levy AR<sup>6</sup>  
<sup>1</sup>Oxford Outcomes Ltd, Oxford, UK, <sup>2</sup>Oxford Outcomes, Oxford, UK, <sup>3</sup>Bristol-Myers Squibb  
Company, Washington Crossing, PA, USA, <sup>4</sup>Bristol-Myers Squibb Pharmaceuticals, Wallingford,  
CT, USA, <sup>5</sup>Oxford Outcomes Ltd., Morristown, NJ, USA, <sup>6</sup>Oxford Outcomes Ltd., Vancouver, BC,  
Canada

**OBJECTIVES:** The impact of new oncology therapies on OS is often assessed by comparing median OS times in randomised controlled trials. Although this data is usually available even when many patients remain alive at the end of the trial, the survival times of those surviving beyond the median point may not be adequately accounted for in this comparison. In this case study, we discuss the median and the mean OS using data from a recently published randomised trial. **METHODS:** Median OS in the ipilimumab-alone (IPI) and gp100 alone-arms of the trial of IPI in pre-treated metastatic melanoma (MM) patients (Hodi et al., 2010, NEJM) was compared with non-parametric estimates of mean survival (area under digitised Kaplan-Meier survivor function) over four years (maximum follow up 55 months). We reviewed the methods literature and approaches adopted in relevant assessments. **RESULTS:** In this case study, for MM population followed over four years median OS was reached in the control arm at 6.4 months, and at 10.1 months in the IPI alone arm, a difference in medians of 3.7 months. Mean OS (area under the curve) over 4 years was 11.5 months in the control arm and 17.6 months in the IPI alone arm, a difference for IPI of 6.1 months. Though larger than the difference in median OS, this represents a lower bound on the mean OS benefit over the remaining lifetime, since the survival benefit was truncated at the end of the trial. **CONCLUSIONS:** Mean and median OS both have a place in characterizing OS. In this case study, it would appear that mean OS may be more informative in describing the potential benefit of the treatment in patients with MM. Health care decision makers should consider all the available data when assessing the potential benefits offered by new therapies in oncology.

## CN2

### MEASURING PUBLIC PREFERENCES FOR COLORECTAL CANCER SCREENING USING NEW GENOME-BASED NANOTECHNOLOGIES

Fermont JM, Groothuis-Oudshoorn K, Jzerman MJ  
University of Twente, Enschede, The Netherlands

**OBJECTIVES:** Emerging developments in nanomedicine allow the development of genome-based technologies for unobtrusive and individualized screening of colorectal cancer. An example is the nanopill that is currently being developed. The pill collects gastrointestinal fluid and screens DNA for tumour markers. The main objective is to inform further development by determining the public preferences for screening as well as the possible uptake of the nanopill compared to standard CRC screening. **METHODS:** Data was collected through a discrete choice experiment among individuals aged between 50 and 74 years living in the The Netherlands and the UK. A full-profile fractional factorial design with a balanced overlap was implemented. Fourteen random and two fixed choice-tasks with triplets and dual-none response were used. Through an extensive literature search following attributes were included: preparation, technique, sensitivity, specificity, complication rate, and testing frequency. Data were analysed using Hierarchical Bayes analysis and a Multinomial Logit model. **RESULTS:** Thirteen hundred fifty-six respondents completed the questionnaire, from which 884 (65%) passed the consistency test. Most preferred attributes were: technique (pill), preparation (none); sensitivity (100%), specificity (100%), complications (none), and interval (every 5 years). Nanopill was the most preferred screening modality (46%), followed by iFOBT (40%), colonoscopy (2%), and sigmoidoscopy (1%). Eleven percent would choose not to be screened. **CONCLUSIONS:** CRC screening has been implemented in a number of countries using standard screening techniques, like FOBT and virtual colonoscopy. However, current developments in nanomedicine allow the development of new technologies for individualized screening. The expected benefits delivered by the nanopill are an improved screening adherence, earlier diagnosis and an increased test performance. The present study suggests the nanopill to be accepted by the public, which does support further development. However, the study used hypothetical scenarios to describe the nanopill and the results do not guarantee market uptake. Cost-benefit analysis and clinical trials remain mandatory.

## CN3

### VALIDATION STUDY OF THE BASELINE QUALITY OF LIFE AS A PROGNOSTIC INDICATOR OF SURVIVAL: A POOLED ANALYSIS OF INDIVIDUAL PATIENT DATA FROM NCIC CLINICAL TRIALS

Ediebah DE<sup>1</sup>, Quinten C<sup>2</sup>, Coens C<sup>3</sup>, Zikos E<sup>1</sup>, Ringash J<sup>4</sup>, Gotay C<sup>5</sup>, Flechtner HH<sup>6</sup>,  
Osoba D<sup>7</sup>, King M<sup>8</sup>, Cleeland C<sup>9</sup>, Greimel E<sup>10</sup>, Reeve BB<sup>11</sup>, Taphoorn M<sup>12</sup>,  
Schmucker-Von Koch J<sup>13</sup>, Weis J<sup>14</sup>, Bottomley A<sup>3</sup>

<sup>1</sup>European Organisation for Research and Treatment of Cancer Head Quarters, Bruxelles, Belgium, <sup>2</sup>European Center for Disease Prevention and Control, Surveillance and Response Support Epidemiological Methods Unit, Stockholm, Sweden, <sup>3</sup>European Organisation for Research and Treatment of Cancer Head Quarters, Bruxelles, Belgium, <sup>4</sup>The Princess Margaret Hospital, Toronto, QC, Canada, <sup>5</sup>U of British Columbia, Vancouver, BC, Canada, <sup>6</sup>Otto-von-Guericke University Magdeburg, Magdeburg, Germany, <sup>7</sup>Quality of Life Consulting, West Vancouver, BC, Canada, <sup>8</sup>University of Sydney, Sydney, Australia, <sup>9</sup>U.T.M.D. Anderson Cancer Center, Houston, TX, USA, <sup>10</sup>Medical University of Graz, Graz, Austria, <sup>11</sup>University of North Carolina at Chapel Hill, Chapel Hill, NC, USA, <sup>12</sup>VU University Medical Center, department of Neurology, The Hague,

The Netherlands, <sup>13</sup>University of Regensburg, Regensburg, Germany, <sup>14</sup>University of Freiburg, Freiburg, Germany

**OBJECTIVES:** Our aims were to investigate the association between baseline health-related quality of life (HRQOL) scores of the EORTC QLQ-C30 and survival. **METHODS:** We analyzed data from pooled, randomized, controlled trials from National Cancer Institute Canada Clinical Trials Group started between 1991 and 2004, which included survival data from 3635 patients with 8 different cancer sites. Sociodemographic variables were sex (men vs. women) and age (60 vs. > 60), and clinical variables were WHO performance status (0-1 vs. 2-3) and distant metastases (no vs. yes). The prognostic significance of sociodemographic and clinical variables and the 15 QLQ-C30 scales were assessed with Cox proportional hazard models stratified for cancer site. **RESULTS:** In the stratified multivariate model including sociodemographic, clinical, and HRQOL data, the HRQOL parameters of global QOL/health status (hazard ratio [HR] 1.097, 95% CI 1.05-1.14; p<.0001), physical function (0.94, 0.897-0.98; p=0.0010), dyspnoea (1.04, 1.00-1.07; p=0.0120), and appetite loss (1.06, 1.03-1.09; p<.0001) provided significant prognostic information in addition to the sociodemographic and clinical variables. The gain in predictive accuracy of prognosis of overall survival of the four HRQOL parameters over the sociodemographic and clinical characteristics was 3% (Harrell's C-index for sociodemographic and clinical variables = 0.69, and for sociodemographic, clinical, and HRQOL variables = 0.71). The model developed by Quinten et al. 2009 included pain but this was not found to be statistically significant in our model. **CONCLUSIONS:** Our findings suggest that HRQOL scales of the EORTC QLQ-C30 provide prognostic information in addition to that of sociodemographic and clinical measures. This replicates previous findings (Quinten et al., 2009) showing that HRQOL data can help to predict survival in patients with cancer, although the specific HRQOL domains that are predictive may vary. The impact of these findings for clinical management (e.g., in stratification for clinical trials entry or treatment decision making) need additional study.

## CN4

### HETEROGENEITY IN PREDICTING THE FUTURE IMPACT OF TECHNOLOGIES TO CONTROL HEPATOCELLULAR CARCINOMA (HCC): A COMPARISON OF STAKEHOLDER VIEWS FROM EUROPE AND ASIA

Bridges JF<sup>1</sup>, Gallego G<sup>1</sup>, Joy SM<sup>1</sup>, Blauvelt BM<sup>2</sup>

<sup>1</sup>Johns Hopkins Bloomberg School of Public Health, Baltimore, MD, USA, <sup>2</sup>University of Massachusetts, Hadley, MA, USA

**OBJECTIVES:** Hepatocellular carcinoma (HCC) is both common and deadly; yet predicting the future impact of technologies is difficult. We studied opinions about the potential impact of HCC-control technologies over a 5-10 year horizon and compared results from Europe and Asia. **METHODS:** Clinical, policy and patient advocacy stakeholders were purposively sampled equally from Asia and Europe. Opinions about eleven possible technologies were studied using best-worst scaling. Here a balanced incomplete block design (BIBD) generated 11 choice tasks presenting respondents with subsets of five technologies and asking them to assess which might have the most and least impact on HCC control. Assuming sequential best-worst choice, respondents' choices were analyzed using a stratified conditional logistic regression. Heterogeneity was examined by assessing ordinal and cardinal properties using Spearman's Rho and Wald test respectively. **RESULTS:** A total of 160 stakeholders (response rate: 46%) completed the survey and self-identified as having local/regional (30%), national (46%) or international (24%) influence. Overall, respondents saw molecular targeted therapy (p <0.001) and early detection (p <0.001) as having most potential, while surgical techniques (p <0.001) and biopsy-free diagnosis (p <0.001) were viewed negatively. While the ordinal rankings of technologies were similar (Spearman's Rho=0.81, P=0.003), significant differences were found for some technologies across regions – e.g. interventional radiology was positively valued in Europe (P=0.002), but viewed negatively in Asia (P=0.118), but adjuvant/neo-adjuvant therapy was viewed positively in Asia (P<0.001), but negatively in Europe (P=0.001). **CONCLUSIONS:** While best-worst scaling methods are likely to have an important role in informing horizon scanning and other aspects of health technology assessment, issues of regional heterogeneity are important to explore. Our results indicated that heterogeneity may be more important when considering the cardinal values placed on the elements being examined, as opposed to the ordinal rankings; heterogeneity was not found for either the best or worst technologies.

## PODIUM SESSION I:

### NEW APPROACHES FOR EFFECTIVE USE OF DATA: BETTER SYNTHESIS AND ENHANCED POWER

## DA1

### ARTIFICIAL NEURAL NETWORK META-MODELS IN COST-EFFECTIVENESS ANALYSIS OF INTENSIVE BLOOD-GLUCOSE CONTROL: A CASE STUDY APPLIED TO THE UK PROSPECTIVE DIABETES STUDY (UKPDS) INDIVIDUAL PATIENT OUTCOME SIMULATION MODEL

Alam M<sup>1</sup>, Briggs A<sup>2</sup>

<sup>1</sup>University of Glamorgan, Pontypridd, UK, <sup>2</sup>Glasgow University, Glasgow, UK

**OBJECTIVES:** Within cost effectiveness analysis, joint uncertainty in costs and effects is commonly dealt with using probabilistic sensitivity analysis (PSA). Although economic models using patient level data can simulate more complex disease processes than cohort-based models, the computational time required to eliminate 1st-order uncertainty often makes extensive PSA impossible. To overcome this, a non-parametric artificial neural network (ANN) simulation meta-modelling method is presented using a case study that evaluates the cost-effectiveness of intensive blood-glucose monitoring in patients with type 2 diabetes. **METHODS:**